

BETH C. DRAIN, CA CSR NO. 7152

BEFORE THE
INDEPENDENT CITIZENS' OVERSIGHT COMMITTEE
AND THE APPLICATION REVIEW SUBCOMMITTEE
TO THE
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
ORGANIZED PURSUANT TO THE
CALIFORNIA STEM CELL RESEARCH AND CURES ACT
REGULAR MEETING

LOCATION: CALIFORNIA INSTITUTE FOR
REGENERATIVE MEDICINE
1999 HARRISON STREET, SUITE 1650
OAKLAND, CALIFORNIA

DATE: OCTOBER 11, 2018
12 P.M.

REPORTER: BETH C. DRAIN, CSR
CA CSR. NO. 7152

FILE NO.: 2018-13A

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I N D E X

ITEM DESCRIPTION	PAGE NO.
OPEN SESSION	
1. CALL TO ORDER.	3
2. ROLL CALL.	3
3. CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO PARTNERING OPPORTUNITY: QUEST AWARDS.	4
CLOSED SESSION	NONE
4. DISCUSSION OF CONFIDENTIAL INTELLECTUAL PROPERTY OR WORK PRODUCT, PREPUBLICATION DATA, FINANCIAL INFORMATION, CONFIDENTIAL SCIENTIFIC RESEARCH OR DATA, AND OTHER PROPRIETARY INFORMATION RELATING TO APPLICATIONS SUBMITTED IN RESPONSE TO AGENDA ITEMS "4" AND "5" ABOVE. (HEALTH & SAFETY CODE 125290.30(F) (3) (B) AND (C)).	
5. PUBLIC COMMENT.	NONE
6. ADJOURNMENT.	35

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THURSDAY, OCTOBER 11, 2018; 12:00 P.M.

CHAIRMAN THOMAS: WELCOME, EVERYBODY, TO
THE REGULAR MEETING OF THE ICOC AND THE APPLICATION
REVIEW SUBCOMMITTEE FOR OCTOBER 2018. MARIA, PLEASE
CALL THE ROLL.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.
DAVID HIGGINS.

DR. HIGGINS: PRESENT.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: HERE.

MS. BONNEVILLE: SHERRY LANSING. DAVE
MARTIN.

DR. MARTIN: HERE.

MS. BONNEVILLE: LAUREN MILLER.

MS. MILLER: HERE.

MS. BONNEVILLE: ADRIANA PADILLA.

DR. PADILLA: HERE.

MS. BONNEVILLE: JOE PANETTA. FRANCISCO
PRIETO. ROBERT QUINT. FRANCISCO PRIETO.

DR. PRIETO: HERE.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: HERE.

MS. BONNEVILLE: JEFF SHEEHY.

MR. SHEEHY: HERE.

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1 MS. BONNEVILLE: OS STEWARD.

2 DR. STEWARD: HERE.

3 MS. BONNEVILLE: JONATHAN THOMAS.

4 CHAIRMAN THOMAS: HERE.

5 MS. BONNEVILLE: ART TORRES.

6 MR. TORRES: HERE.

7 MS. BONNEVILLE: DIANE WINOKUR.

8 EVERYONE, WE ARE JUST AT QUORUM. SO
9 PLEASE STAY ON FOR THE LENGTH OF THE MEETING. THANK
10 YOU SO MUCH.

11 CHAIRMAN THOMAS: WE'RE GOING TO GO
12 DIRECTLY TO ITEM 3, CONSIDERATION OF APPLICATIONS
13 SUBMITTED IN RESPONSE TO THE PARTNERING OPPORTUNITY,
14 QUEST AWARDS. AND I'LL TURN THE MEETING NOW OVER TO
15 MR. SHEEHY.

16 MR. SHEEHY: THANK YOU, CHAIRMAN THOMAS.
17 DR. SAMBRANO HAS A PRESENTATION FOR US.

18 DR. SAMBRANO: YES. THANK YOU,
19 MR. SHEEHY.

20 SO I HAVE A SLIDE PRESENTATION THAT WAS
21 DISTRIBUTED AND ALSO SHOWING ON WEBEX. SO THIS IS
22 BASICALLY A REMINDER OF WHERE WE LEFT OFF ON THE
23 QUEST RECOMMENDATIONS FROM THE GWG FOR THIS PROGRAM.
24 SO JUST A REMINDER OF WHAT THE QUEST PROGRAM IS
25 ABOUT. THIS IS BASICALLY OUR DISCOVERY ENGINE

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1 PROGRAM THAT TAKES PROMISING NEW STEM CELL-BASED
2 TECHNOLOGIES TO THE POINT WHERE THEY CAN BEGIN
3 TRANSLATIONAL STUDIES WITHIN A PERIOD OF ABOUT TWO
4 YEARS.

5 WHEN WE PRESENTED THIS IN JULY, THE
6 RECOMMENDATIONS FROM THE GRANTS WORKING GROUP WERE
7 SHOWN ON THE TABLE. SO WE HAD 14 APPLICATIONS FOR
8 WHICH THERE WAS A TOTAL REQUEST ASK OF ABOUT \$19
9 MILLION. HOWEVER, THE FUNDS AVAILABLE OR ALLOCATED
10 FOR THIS PARTICULAR CYCLE WAS 10 MILLION. SO THE
11 AMOUNT REQUESTED WAS CERTAINLY WELL OVER THE 10
12 MILLION.

13 WHAT HAPPENED THEN IN THE JULY 19 ICOC/ARS
14 MEETING WAS THAT THIS COMMITTEE APPROVED FUNDING OF
15 \$10 MILLION FOR EIGHT DISC2 APPLICATIONS THAT
16 INCLUDED PARTIAL FUNDING FOR TWO OF THEM. AND THEN
17 THE REMAINING SIX APPLICATIONS THAT WERE RECOMMENDED
18 BY THE GRANTS WORKING GROUP WERE DEFERRED IN TERMS
19 OF MAKING A DECISION WITH THE POSSIBILITY OF
20 ALLOCATING FUTURE FUNDS TO THOSE.

21 SUBSEQUENT TO THAT MEETING, CIRM RECOVERED
22 ABOUT \$865,000 FROM ONE OF THOSE APPLICATIONS. IT
23 WAS THE DISC2-11192. WHAT HAPPENED THERE WAS THE
24 PROJECT PI MOVED OUT OF STATE AND SO THE FUNDS WERE
25 NOT AWARDED. THIS IS ONE OF THE PARTIALLY FUNDED

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1 APPLICATIONS. AND SO WE RECOVERED THAT AMOUNT.

2 AND SO AS A CONSEQUENCE WHAT WE ARE
3 RECOMMENDING IN TERMS OF ACTIONS MOVING FORWARD IS
4 TO FULLY FUND THE OTHER PARTIALLY FUNDED
5 APPLICATION, WHICH IS DISC2-11109. THAT WOULD
6 UTILIZE ABOUT 550,000 OF THOSE AVAILABLE NOW 865,000
7 THAT WE RECOVERED.

8 AND THEN THE OTHER RECOMMENDATION WOULD BE
9 NOT TO FUND THE REMAINING SIX RECOMMENDED DISC2
10 APPLICATIONS IN LIEU OF THE BUDGET. AND THOSE ARE
11 OUR RECOMMENDATIONS, MR. SHEEHY.

12 MR. SHEEHY: THANK YOU.

13 SO FIRST OF ALL, DO WE -- WE'LL KIND OF
14 BREAK THIS UP. DO WE HAVE A MOTION TO ACCEPT THE
15 FIRST RECOMMENDATION, WHICH IS TO PARTIALLY FUND --
16 FULLY FUND THE PARTIALLY FUNDED APPLICATION
17 QUEST2-11109?

18 DR. JUELSGAARD: SO MOVED.

19 MR. SHEEHY: IS THERE A SECOND?

20 DR. PRIETO: I'LL SECOND.

21 MR. SHEEHY: IS THERE ANY DISCUSSION? IS
22 THERE ANY PUBLIC COMMENT ON THIS MOTION EITHER HERE
23 OR AT ANY OF THE SITES?

24 WE'RE GOING TO TALK ABOUT THE REST OF THE
25 APPLICATIONS WITH SOME SORT OF MOTION FOLLOWING.

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1 MR. REED: SO THE 865,000 IS NOT INCLUDED
2 IN WHAT YOU ARE SAYING? JUST FOR THE RECORD, IF THE
3 865,000 WAS INCLUDED, HE SAYS IT'S NOT.

4 MR. SHEEHY: FIVE FIFTY IS INCLUDED TO
5 FULLY FUND THE APPLICATION WE ONLY FUNDED HALF OF.

6 MR. REED: THE 865,000 -- THEN DO NOT
7 REFUND THE REMAINING, WE'RE NOT TALKING ABOUT THAT
8 YET?

9 MR. SHEEHY: YES, WE ARE. WE'RE TALKING
10 ONLY ABOUT THE FIRST TEAM RECOMMENDATION, WHICH IS
11 TO TAKE -- WE GOT \$865,000 BACK -- TO TAKE ABOUT 550
12 OF THAT AND TO FULLY FUND AN APPLICATION THAT WE
13 PARTIALLY FUNDED AT THE LAST MEETING.

14 MR. REED: SO WHAT WE WOULD BE DISCUSSING
15 RIGHT NOW IS THAT THERE'S NO WAY TO FUND THE
16 REMAINING GRANTS?

17 MR. SHEEHY: WE'LL TALK ABOUT THE
18 REMAINING GRANTS WITH ANOTHER MOTION, WITH A SECOND
19 MOTION.

20 MR. REED: NOW IS NOT THE TIME TO DISCUSS
21 THAT?

22 MR. SHEEHY: NO. THANKS, DON. ANY OTHER
23 PUBLIC COMMENT? COULD WE CALL THE ROLL.

24 MS. BONNEVILLE: ANNEMARIE DULIEGE. DAVID
25 HIGGINS.

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1 DR. HIGGINS: YES.
2 MS. BONNEVILLE: STEVE JUELSGAARD.
3 MR. JUELSGAARD: YES.
4 MS. BONNEVILLE: SHERRY LANSING. DAVE
5 MARTIN.
6 DR. MARTIN: YES.
7 MS. BONNEVILLE: LAUREN MILLER.
8 MS. MILLER: YES.
9 MS. BONNEVILLE: ADRIANA PADILLA.
10 DR. PADILLA: YES.
11 MS. BONNEVILLE: JOE PANETTA. FRANCISCO
12 PRIETO.
13 DR. PRIETO: AYE.
14 MS. BONNEVILLE: ROBERT QUINT. AL
15 ROWLETT.
16 MR. ROWLETT: YES.
17 MS. BONNEVILLE: JEFF SHEEHY.
18 MR. SHEEHY: YES.
19 MS. BONNEVILLE: OS STEWARD. JONATHAN
20 THOMAS.
21 CHAIRMAN THOMAS: YES.
22 MS. BONNEVILLE: ART TORRES.
23 MR. TORRES: AYE.
24 MS. BONNEVILLE: OS, I THINK YOU'RE ON
25 MUTE.

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1 DR. STEWARD: YEAH. ACTUALLY I WAS ON THE
2 OTHER PHONE WHICH APPARENTLY PUT ITSELF ON MUTE. SO
3 THE ANSWER IS YES.

4 MS. BONNEVILLE: MOTION CARRIES.

5 MR. SHEEHY: THANK YOU. OKAY. SO WE NEED
6 TO COME TO SOME SORT OF CONCLUSION ABOUT THE
7 REMAINING SIX RECOMMENDED APPLICATIONS. THE TEAM
8 RECOMMENDED NOT FUNDING THEM. I KNOW THAT THERE IS
9 AN OPTION --

10 CHAIRMAN THOMAS: J.T. WITH RESPECT TO
11 THAT, TO GET THE CONVERSATION GOING HERE, I'D LIKE
12 TO MAKE A MOTION WITH RESPECT TO THE REMAINING
13 AWARDS, WHICH IS I MOVE THAT WE CONTINUE
14 CONSIDERATION OF THESE AWARDS TO A FUTURE MEETING OF
15 THE APPLICATION REVIEW SUBCOMMITTEE IN THE EVENT THE
16 ICOC APPROVES THE USE OF BRIDGE FUNDS TO FUND THESE
17 AWARDS.

18 MR. TORRES: SECOND.

19 MR. SHEEHY: SECOND BY ART. DO WE HAVE
20 DISCUSSION? WE HAD TALKED AT A PREVIOUS MEETING
21 ABOUT PUTTING IT OFF. SO WHAT ARE PEOPLE'S THOUGHTS
22 ON THIS?

23 DR. JUELSGAARD: WE DON'T HAVE THE MONEY
24 AT THIS POINT. SO IT'S OVER WITH, RIGHT? I DON'T
25 KNOW WHAT DISCUSSION CAN BE HAD AT THIS POINT.

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1 MR. SHEEHY: OKAY. IS THERE ANY OTHER
2 COMMENT BY ANY BOARD MEMBERS OR QUESTIONS?

3 DR. PRIETO: MR. SHEEHY, MR. CHAIRMAN,
4 THIS IS FRANCISCO PRIETO. SO QUESTION. IF WE
5 ACCEPT THIS MOTION, DOES THAT JUST KEEP THESE
6 APPLICATIONS ALIVE AS OPPOSED TO REJECTING THEM SO
7 THAT WHEN FUNDS BECOME AVAILABLE OR IN THE NEXT
8 FISCAL YEAR, WE COULD THEN SIMPLY VOTE ON THEM
9 BECAUSE THEY'VE ALREADY BEEN APPROVED BY THE GWG?

10 MR. SHEEHY: THIS DOES KEEP THE
11 APPLICATION ALIVE, BUT SOLELY FOR BRIDGE FUNDING.

12 DR. PRIETO: OKAY. THANK YOU.

13 MR. SHEEHY: MAYBE WE SHOULD ASK SOME
14 CLARIFICATION ABOUT BRIDGE FUNDING.

15 DR. JUELGAARD: JEFF, BEFORE WE GET INTO
16 THAT, THE WHOLE IDEA OF WHAT WE'RE GOING TO DO WITH
17 OUR MONEY NEXT YEAR IS A SUBJECT YET TO BE BROACHED.
18 I KNOW WHAT THE RECOMMENDATIONS ARE GOING TO BE, BUT
19 UNTIL WE HAVE AN ACTUAL ICOC MEETING AND MAKE THAT
20 DETERMINATION, THIS IS A PLAN AT THIS TIME. THAT'S
21 WHAT I UNDERSTAND.

22 CHAIRMAN THOMAS: THAT'S CORRECT, STEVE.

23 MR. SHEEHY: SO IS THERE ADDITIONAL
24 DISCUSSION?

25 MR. ROWLETT: I DON'T HAVE ANY OBJECTION

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1 TO THE POSSIBILITY OF A DISCUSSION ASSOCIATED WITH
2 BRIDGE FUNDING. THE OUTCOME OF THESE APPLICATIONS
3 COULD BE THE SAME AFTER THAT DISCUSSION IF YOU
4 CHOOSE NOT TO DO BRIDGE FUNDING. SO I DON'T HAVE
5 ANY OBJECTION TO IT.

6 MR. SHEEHY: A DISCUSSION OF BRIDGE
7 FUNDING?

8 CHAIRMAN THOMAS: NOT A LOT TO DISCUSS IN
9 THIS FORUM OTHER THAN WE ARE BUSILY OUT LOOKING TO
10 RAISE THAT WITH A NUMBER OF PEOPLE. I DON'T REALLY
11 WANT TO HAVE A REPORT ON THAT BECAUSE THIS ISN'T
12 SORT OF THE TIME. I'LL GET INTO THAT MORE AT OUR
13 BOARD MEETING NEXT WEEK.

14 MR. SHEEHY: OKAY. SO WE HAVE A MOTION.
15 IS THERE FURTHER BOARD DISCUSSION? THEN I OPEN UP
16 TO PUBLIC COMMENT.

17 MR. REED: I JUST THINK IT'S IMPORTANT
18 THAT WE KEEP THESE GRANTS ALIVE AND OPEN FOR WHEN
19 THERE WILL BE, I BELIEVE, SOME MONEY COMING IN. I
20 JUST HAVE A BELIEF THAT THERE WILL BE SOME MONEY
21 COMING IN. I HAVE FAITH IN J.T., I HAVE FAITH IN A
22 LOT OF PEOPLE. I THINK WE WILL GET SOME MORE MONEY.
23 I DON'T WANT TO SEE THESE SIX OUTSTANDING
24 PROGRAMS -- ALL OF THEM ARE GOOD. I HAVE MY
25 FAVORITE, BUT THEY'RE ALL GOOD -- I DON'T WANT TO

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1 SEE THEM CUT OFF. SO I THINK IT'S IMPORTANT THAT WE
2 LEAVE THEM OPEN FOR THAT.

3 MR. SHEEHY: WE HAVE ANOTHER PUBLIC
4 COMMENT HERE IN SAN FRANCISCO.

5 DR. BEACHY: GOOD AFTERNOON, EVERYONE.
6 I'M PHIL BEACHY. I'M A PROFESSOR AT STANFORD
7 UNIVERSITY MEDICAL CENTER. I HAVE APPOINTMENTS IN
8 DEPARTMENTS OF UROLOGY, DEVELOPMENTAL BIOLOGY,
9 CHEMICAL AND SYSTEMS BIOLOGY, AND, OF COURSE, I'M A
10 MEMBER OF THE STANFORD STEM CELL BIOLOGY INSTITUTE,
11 AS WELL AS THE CANCER INSTITUTE.

12 JUST BY WAY OF BRIEF INTRODUCTION, I HAVE
13 SOME EXPERIENCE WITH DRUG DEVELOPMENT. I HAVE BEEN
14 A CO-FOUNDER OF THREE CALIFORNIA BIOTECHS. ONE OF
15 THESE IS (INAUDIBLE), ANOTHER IS IN PHASE 3 TRIALS
16 NOW, AND ANOTHER ONE ACTUALLY HAS BEEN PUBLICLY
17 TRADED SINCE 2013.

18 THE REASON WE'RE HERE TODAY IS BECAUSE OUR
19 TEAM SUBMITTED A PROPOSAL ON USE OF EMBRYONIC STEM
20 CELLS TO PROVIDE A DEFINITIVE, LONG-TERM CARE FOR
21 BLADDER CANCER. AND THAT'S WHAT WE WANT TO TALK
22 ABOUT.

23 BLADDER CANCER IS THE FIRST MOST COMMON
24 CANCER IN MEN. IT ALSO OCCURS IN WOMEN. BUT CIRM
25 HAS NOT PREVIOUSLY FUNDED ANY WORK ON BLADDER

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1 CANCER. NOW, THE SCIENTIFIC PANEL RANKED OUR
2 PROPOSAL IN A TIE FOR THIRD PLACE AMONG 41
3 APPLICATIONS, BUT OUR APPLICATION HAS NOT YET BEEN
4 FUNDED. WE SUBSEQUENTLY LEARNED THAT AN IMPORTANT
5 PART OF OUR PROPOSAL IS TO PRESENT IT TO THIS
6 COMMITTEE. AND SO WE'RE GRATEFUL TO HAVE A CHANCE
7 TO DO THAT TODAY.

8 THE MAJOR PROBLEM WITH BLADDER CANCER IS
9 THAT IT OFTEN RETURNS, EVEN AFTER THE PRIMARY TUMOR
10 IS REMOVED. AND THIS MAKES BLADDER CANCER VERY
11 RISKY TO HAVE, AND ALSO VERY DIFFICULT TO TREAT
12 BECAUSE EVEN AFTER SUCCESSFUL TUMOR REMOVAL, THE
13 PATIENT HAS TO BE CONTINUOUSLY MONITORED, AND THIS
14 MAKES IT THE MOST EXPENSIVE CANCER TO TREAT PER
15 PATIENT OF ANY CANCER.

16 NOW, IN MY LAB'S RESEARCH, WE LEARNED THAT
17 THE CANCER RETURNS BECAUSE THE ENTIRE LINING OF THE
18 BLADDER THAT REMAINS AFTER TUMOR REMOVAL COMES FROM
19 THE SAME INITIAL STEM CELL THAT GAVE RISE TO THE
20 TUMOR. AND THIS CORRUPTIVE BLADDER LINING THEN HAS
21 SOME OF THE CHANGES ALREADY PRESENT THAT GIVE RISE
22 TO THE TUMOR, AND THIS MAKES IT ACTUALLY MUCH EASIER
23 FOR THE TUMOR TO RETURN AFTER EXCISION OF THE
24 PRIMARY TUMOR.

25 SO THE ONLY WAY TO REALLY SOLVE THIS

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1 PROBLEM IS TO GET RID OF THE CORRUPTED LINING, AND
2 THAT'S EXACTLY WHAT WE PROPOSE TO DO. WE PLAN TO
3 REMOVE THE OLD LINING WITH A SPECIAL DRUG AND
4 REPLACE IT WITH PRISTINE LINING CELLS THAT ARE
5 DERIVED FROM EMBRYONIC STEM CELLS. THIS SHOULD
6 PROVIDE A PERMANENT SOLUTION AND REDUCE RISK AND
7 REDUCE EXPENSE OF BLADDER CANCER TREATMENT.

8 TO HELP US DO THIS, WE'VE ASSEMBLED A TEAM
9 THAT CONSISTS OF DR. JOE LIAO, WHO'S A BLADDER
10 CANCER SPECIALIST AND A UROLOGIST AT STANFORD.
11 HE'LL SPEAK TO US IN A MINUTE. AND DR. LAY TENG ANG
12 AND DR. KYLE LOH, BOTH OF WHOM ARE REAL WHIZZES AT
13 GETTING EMBRYONIC STEM CELLS TO DIFFERENTIATE AND TO
14 MAKE WHATEVER KINDS OF CELLS ARE NEEDED.

15 I'LL JUST CLOSE QUICKLY BY SAYING THAT
16 I'LL TURN IT OVER TO MY COLLEAGUES HERE, AND WE HOPE
17 THAT YOU WILL CONSIDER OUR APPLICATION FOR FUNDING.
18 THANK YOU.

19 MR. SHEEHY: YOU CAN BORROW TIME FROM ONE
20 OR TWO OF YOUR COLLEAGUES IF YOU'D LIKE TO DO THAT.

21 DR. BEACHY: ESSENTIALLY I JUST WANTED TO
22 INTRODUCE MY COLLEAGUES. I'VE ALREADY INTRODUCED
23 JOE AND KYLE AND LAY TENG. KYLE IS ALSO A PROFESSOR
24 AT STANFORD IN THE STEM CELL INSTITUTE, AND LAY TENG
25 IS AN INDEPENDENT RESEARCH FELLOW. SO THANK YOU.

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1 DR. LIAO: THANK YOU, PHIL. MY NAME IS
2 JOE LIAO. I'M A PROFESSOR OF UROLOGY AT STANFORD
3 SCHOOL OF MEDICINE, AND I ALSO SERVE AS THE CHIEF OF
4 UROLOGY AT THE PALO ALTO VA HOSPITAL.

5 SO AS A CANCER SURGEON AND A RESEARCHER,
6 I'VE BEEN COLLABORATING WITH DR. BEACHY FOR THE PAST
7 FOUR YEARS. I VERY MUCH SHARE HIS ENTHUSIASM TO BE
8 HERE TODAY TO DESCRIBE TO YOU OUR RESEARCH MISSION
9 TOWARD FINDING A DEFINITIVE CURE FOR BLADDER CANCER.

10 IN MY CLINICAL PRACTICE I TAKE CARE OF A
11 LARGE NUMBER OF BLADDER CANCER PATIENTS ACROSS
12 DIFFERENT STAGES. AT AN EARLY STAGE, THE TUMOR CAN
13 TYPICALLY BE RESECTED THROUGH A CAMERA AND AN
14 ENDOSCOPE. HOWEVER, AS DR. BEACHY MENTIONED, THE
15 RECURRENCE RATE OF BLADDER CANCER IS VERY, VERY
16 HIGH, AND THE MAJORITY OF THE CANCER WILL INDEED
17 RECUR. PATIENTS HAVE TO UNDERGO FREQUENT INVASIVE
18 ENDOSCOPY PROCEDURES CALLED CYSTOSCOPIES AND
19 FREQUENT TRIPS BACK TO THE OPERATING ROOM WHEN THEY
20 DO RECUR.

21 OUR RESEARCH HAS SHOWN THAT THE HIGH
22 RECURRENCE RATE IS IN LARGE PART DUE TO THE
23 PERSISTENCE OF CORRUPTED BLADDER LINING EVEN AFTER
24 SURGERIES. ONE OF THE TREATMENT OPTIONS WE HAVE IS
25 CALLED BCG IN WHICH WE INTRODUCE A LOW-GRADE

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1 INFECTION INTO THE BLADDER IN THE EFFORT TO REV UP
2 THE IMMUNE SYSTEM TO REDUCE THE CHANCE OF CANCER
3 RETURNING. HOWEVER, THE RESPONSE RATE OF BCG IS
4 SUBOPTIMAL. AND IF A PATIENT WERE TO CONTINUE TO
5 HAVE RECURRENCE DESPITE BEING ON BCG, THEN HE OR SHE
6 IS FACED WITH THE POSSIBILITY OF REMOVAL OF THE
7 ENTIRE BLADDER AND RECONSTRUCTION OF A NEW BLADDER
8 USING INTESTINAL SEGMENTS. AND THIS IS A HIGHLY
9 CHALLENGING AND COMPLICATED SURGERY WHERE
10 ESSENTIALLY WE ARE COMPLETELY CHANGING THE PLUMBING
11 SYSTEM, IF YOU WILL, FOR THE PARTICULAR PATIENT.
12 OUR GOAL IS TO CHANGE THAT TREATMENT PARADIGM.

13 I WILL INTRODUCE YOU NEXT TO A PATIENT OF
14 MINE FROM THE VA. VERNON BROWN IS A VETERAN AND A
15 RETIRED DENTIST. HE HAS GRACIOUSLY AGREED TO SHARE
16 WITH YOU SOME OF HIS STORY AND JOURNEY AND BATTLE
17 WITH BLADDER CANCER. UNFORTUNATELY HE DOES HAVE
18 RECURRENT, HIGH-RISK BLADDER CANCER AND HAS HAD
19 INTESTINAL RESECTIONS AS WELL AS BCG TREATMENT.

20 AND HE'S EXACTLY THE TYPE OF PATIENT, THE
21 TYPE OF RESEARCH THAT WE'RE PROPOSING WE'RE LIKELY
22 TO HELP. AND IT IS MY FIRM BELIEF THAT THE BEST
23 BLADDER IS THE ONE THAT YOU WERE BORN WITH, AND THAT
24 IS OUR INTENT THROUGH THIS RESEARCH PROJECT. I'M
25 GOING TO TURN IT OVER TO VERNON NOW. I'LL KEEP

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1 JOINING ON A LINE FROM MONTEREY.

2 DR. BROWN: HELLO. YES. I'M VERNON
3 BROWN, AND I'M 78 YEARS OLD AND I'M A RETIRED
4 DENTIST AND ALSO A VETERAN OF THE VIETNAM WAR. I
5 LIVE IN MONTEREY, AND I PRACTICED IN MONTEREY FOR
6 ABOUT 34 YEARS AFTER LEAVING THE MILITARY.

7 I WAS DIAGNOSED WITH BLADDER CANCER IN
8 EARLY 2017. IT SHOWED UP IN A CT SCAN THAT I WAS
9 HAVING FOR PROSTATE CANCER. AND I WAS LUCKY IT WAS
10 FOUND BECAUSE I HAD NO SYMPTOMS AND NO PROBLEMS WITH
11 MY BLADDER AT THAT POINT. I DID HAVE A FRIEND WHO
12 JUST RECENTLY DIED OF BLADDER CANCER. HE WAS NOT SO
13 LUCKY.

14 SO, ANYHOW, I HAD THE CYSTOSCOPY OF WHERE
15 THEY FOUND THE LESION. IT'S A SCOPE THAT GOES UP
16 INTO THE BLADDER AND SHOWS WHERE THE CANCER IS.
17 THEN I HAD A SEPARATE APPOINTMENT DONE FOR A BIOPSY
18 OF THE LESION BY ENDOSCOPIC PROCEDURE UNDER GENERAL
19 ANESTHESIA. THE PATHOLOGY REPORT SHOWED,
20 FORTUNATELY, THAT THE CANCER DID NOT GO INTO THE
21 MUSCLE OF THE BLADDER, BUT IT WAS STILL A HIGH LEVEL
22 CANCER.

23 SO AFTER HEALING FROM THAT PROCEDURE, I
24 HAD ANOTHER BIOPSY -- I HAD A PROCEDURE WHERE THEY
25 WENT FOR FOLLOW-UP TO GET THE REST OF THE CANCER

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1 OUT, AND THAT WAS DONE ALSO BY ENDOSCOPIC PROCEDURE.
2 THERE WAS A LONG TIME HEALING FROM THAT, A LOT OF
3 BLEEDING, A LOT OF BLOOD CLOTS. TOOK A LONG TIME TO
4 HEAL. AFTER THAT, I FINALLY WENT INTO THE BCG
5 TREATMENTS WHICH TOOK ABOUT SIX WEEKS, ONE TREATMENT
6 A WEEK.

7 SO THAT LEAVES ME ABOUT WHERE I AM RIGHT
8 NOW. AND MY NEXT EXAMINATION IS NEXT WEEK WITH
9 ANOTHER CYSTOSCOPY. AND THIS CANCER COMES BACK, SO
10 I'M GOING TO FACE A LOT OF TREATMENTS OR FOLLOW-UP
11 TREATMENTS WITH CYSTOSCOPIES AND BCG TREATMENTS AND
12 MOST LIKELY FOR THE REST OF MY LIFE.

13 THIS IS SOMETHING THAT I HAVE TO FACE AND
14 SOMETHING THAT ALL OF THE OTHER PEOPLE THAT HAVE
15 BLADDER CANCER HAVE TO FACE. SO I'M REALLY HOPING
16 FOR A DEFINITIVE CURE THAT WILL HELP ME AND HELP
17 OTHERS WHO HAVE BLADDER CANCER.

18 I THINK DR. BEACHY AND DR. LIAO'S RESEARCH
19 FOR THIS CURE WILL BE VERY, VERY HOPEFUL. SO THANK
20 YOU.

21 DR. BEACHY: THANK YOU, VERNON. THANK YOU
22 SO MUCH.

23 DR. LOH: GOOD AFTERNOON. MY NAME IS KYLE
24 LOH. I'M ASSISTANT PROFESSOR AT THE STANFORD CANCER
25 INSTITUTE. I'M WITH MY COLLEAGUES HERE TODAY TO

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1 DISCUSS OUR PROPOSAL, DISC2-11105, TO USE EMBRYONIC
2 STEM CELL TECHNOLOGY TO DEFINITELY CURE BLADDER
3 CANCER.

4 SO TO PROVIDE SOME CONTEXT, EMBRYONIC STEM
5 CELLS HAVE BEEN MY ABIDING LOVE SINCE I WAS 14 WHEN
6 I BEGAN COLLEGE AND WORKED IN A STEM CELL RESEARCH
7 LAB. AS YOU KNOW, ONE OF CIRM'S MISSIONS IS TO MAKE
8 EMBRYONIC STEM CELL THERAPIES A REALITY. EMBRYONIC
9 STEM CELLS ARE REMARKABLE CELLS BECAUSE THEY CAN
10 TURN INTO ALL THE DIFFERENT KINDS OF CELLS IN THE
11 HUMAN BODY. HOWEVER, AS THEY SAY, IN LIFE IT'S NOT
12 A GOOD THING TO HAVE TOO MANY CHOICES, AS YOU CAN
13 SEE BY TODAY'S DISCUSSION. IT IS CERTAINLY THE CASE
14 FOR EMBRYONIC STEM CELLS. BECAUSE THEY CAN TURN
15 INTO ALL THE DIFFERENT CELL TYPES OF THE BODY, IT'S
16 VERY DIFFICULT TO EFFICIENTLY TURN THEM INTO A
17 SINGLE KIND OF CELL.

18 IT IS EXACTLY WHAT OUR CIRM QUEST TEAM HAS
19 SUCCEEDED IN. SO BACKED BY PREVIOUS CIRM TOOLS AND
20 TECHNOLOGIES AND QUEST AWARDS, WE'VE SUCCEEDED IN
21 TURNING, FOR THE FIRST TIME, HUMAN EMBRYONIC STEM
22 CELLS INTO PURE BATCHES OF LIVER, BONE, AND HEART
23 CELLS. THAT WAS REPORTED IN JOURNAL OF *CELL* AND
24 *CELL STEM CELL* WITH CIRM SUPPORT.

25 NOW WE'VE SUCCEEDED IN CREATING, FOR THE

BETH C. DRAIN, CA CSR NO. 7152

1 FIRST TIME, PURE BATCHES OF HUMAN BLADDER CELLS IN A
2 DISH FROM EMBRYONIC STEM CELLS. AND OUR MISSION IS
3 TO THEN INJECT THESE NEW HUMAN BLADDER CELLS INTO
4 PATIENTS WITH BLADDER CANCER TO REGENERATE THEIR
5 HEALTHY BLADDER LINING.

6 SO HAVING WORKED WITH CIRM BEFORE, I KNOW
7 OF CIRM'S URGENCY TO ACCELERATE STEM CELL TREATMENTS
8 TO PATIENTS WITH UNMET MEDICAL NEEDS. AS YOU JUST
9 HEARD FROM VERNON AND FROM JOE, TREATING BLADDER
10 CANCER IS CERTAINLY AN UNMET MEDICAL NEED. THE
11 PROCEDURES THEY TALKED ABOUT TODAY ARE BASICALLY
12 GIVING PATIENTS A BACTERIAL INFECTION TO BCG, GIVING
13 THEM REGULAR MONITORING, AND EVENTUALLY POTENTIALLY
14 REMOVING THEIR ENTIRE BLADDER.

15 SO OUR TEAM HAS THE SPEED AND THE
16 CAPABILITY AND THE SCIENTIFIC EXCELLENCE TO REALLY
17 MAKE THIS TREATMENT A REALITY. AND I'M HERE TO ASK
18 YOUR HELP TO DO SO. THANK YOU.

19 DR. LAY TENG ANG: MY NAME IS LAY TENG
20 ANG. I'M AN INVESTIGATOR AND INSTRUCTOR AT STANFORD
21 STEM CELL INSTITUTE. TODAY I WOULD LIKE TO SHARE
22 WITH YOU ON A MORE PERSONAL NOTE OF MY INTEREST AND
23 WHY I'M INTERESTED -- WHY I'M MOTIVATED TO WORK
24 EMBRYONIC STEM CELLS.

25 WHEN I WAS 14, MY AUNT SUDDENLY PASSED

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1 AWAY FROM KIDNEY FAILURE. AND SINCE THEN I HAVE
2 COMMITTED MY RESEARCH TO WORK ON STUDIES THAT HAVE
3 THERAPEUTIC POTENTIAL BECAUSE MY LONG-TERM GOAL IS
4 TO TURN EMBRYONIC STEM CELLS INTO HIGHLY USEFUL AND
5 THERAPEUTICALLY USEFUL TISSUE CELL TYPES.

6 OVER THE PAST DECADE, I HAVE DEDICATED MY
7 RESEARCH TO GENERATE HUMAN LIVER AND BLADDER CELLS
8 FROM EMBRYONIC STEM CELLS. I RECENTLY CAME TO
9 STANFORD FROM SINGAPORE. OVER THE PAST SEVEN YEARS,
10 I'VE COLLABORATED WITH EXCELLENT SCIENTISTS AT THE
11 STANFORD STEM CELL INSTITUTE, INCLUDING PHIL BEACHY,
12 KYLE LOH. AND I'M VERY INSPIRED. THAT'S THE REASON
13 ALSO WHY I'M HERE. I CHOOSE TO BE HERE BECAUSE I
14 BELIEVE, I STRONGLY BELIEVE THAT THIS IS THE PLACE
15 WHERE I CAN TRANSLATE MY RESEARCH AND EXTEND MY
16 RESEARCH TO BRING IT TO PATIENTS.

17 SO FAR WE HAVE BEEN VERY SUCCESSFUL IN
18 TURNING EMBRYONIC STEM CELLS INTO BLADDER CELLS.
19 AND I PLAN TO MAKE MY TIME HERE WORTHWHILE BY GIVING
20 IT MY BEST EFFORT AND PUTTING MY BEST FOOT FORWARD.
21 I WILL BE HAPPY TO TAKE QUESTIONS.

22 MR. KHALID: GOOD MORNING, LADIES AND
23 GENTLEMEN. MY NAME IS ALI. I'M THE FATHER OF THREE
24 YEARS OLD WHO WAS DIAGNOSED WITH A SEVERE GENETIC
25 DISORDER, CHROMOSOME 5, WHICH CONTAINS ONE MILLION

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1 BASE, 6 PERCENT OF TOTAL BRAIN CELLS. THIS IS ONE
2 OF THE FIFTH LARGEST CHROMOSOMES. AND THE LESION IS
3 5 Q PARTIAL DELETION AREA 14.3. AND THE SIZE OF THE
4 LESION IS 1.7 HKB WHICH IS LOCATED IN MEF2C GENE.
5 IT'S A VERY ACTIVE PART OF THE BRAIN WHICH CONTROLS
6 OTHER FORM OF RELATED AUTISM.

7 AND IT'S A KEY TO -- FOR OTHER -- TO TREAT
8 OTHER -- WE ARE VERY HOPEFUL PARENTS THAT THIS IS
9 THE ONLY HOPE WE HAVE, WHICH IS DR. STUART LIPTON.
10 HE'S BEEN WORKING WITH US FOR ABOUT SIX MONTHS. DR.
11 LIPTON IS STUDYING HEAD CELLS MADE FOR MY SON AS A
12 MODEL OF THE DISEASE, AND THIS ALL FOR HOPE TO
13 FINDING A DRUG TO REVERSE THE AFFECTED NERVE. IT
14 ALSO CONTROLS THE OTHER PART OF WHICH ARE IMPORTANT
15 FOR ALL AUTISM KIDS.

16 AS PARENTS WE BRING BRIGHT AND COLORFUL
17 FUTURE FOR OUR SON. EVERY DAY WE SEE HIM GO THROUGH
18 SADNESS, DEPRESSION, AND WE SEE PAIN IN HIS EYES.
19 HE CANNOT EXPRESS HIS FEELINGS. HE CANNOT TELL WHAT
20 HE FEELS EVERY DAY. AND EVERY NIGHT HE WAKES UP AT
21 2 A.M. OR 3 A.M. AND HE CANNOT GO BACK TO SLEEP. HE
22 CANNOT SHUT OFF HIS BRAIN. AND WE SEE HIM LIKE THIS
23 EVERY DAY, AND WE ARE SUFFERING OUR LIVES. IT'S
24 AFFECTING ON THAT. AND THIS IS THE ONLY HOPE WE
25 HAVE.

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1 DR. LIPTON IS DOING SOME RESEARCH ON HIM.
2 AND WE DON'T HAVE A NORMAL LIFE ANYMORE AS A PARENT.
3 WE LOOK ANYWHERE -- EVERYWHERE IS DARK. WE HAVE NO
4 HOPE, NO OTHER PLACE WHERE YOU CAN GO. THIS IS THE
5 ONLY CHANCE WE HAVE IN THIS WORLD THAT CAN HELP CURE
6 MY SON.

7 WE'VE BEEN DEALING WITH THIS FROM LAST
8 THREE YEARS. WE DON'T HAVE A NORMAL LIFE, ME AND MY
9 WIFE. WE HAVE ANOTHER EIGHT YEARS OLD. WE CANNOT
10 GO OUT. WE CANNOT EAT. WE ARE ALL IN DEPRESSION
11 BECAUSE OF THIS DISEASE. IT AFFECT OUR FAMILY
12 REALLY BAD.

13 SO I'M NOT FROM ANY HOSPITAL OR ANY OTHER
14 PLACE. I'M JUST A PARENT, YOU KNOW. AND THIS IS A
15 PRACTICAL BECAUSE I'M SEEING THIS, AND WE ARE
16 FEELING, ME AND MY WIFE AND OUR FAMILY, FEELING THIS
17 AND GOING THROUGH THIS EVERY DAY AND PART OF OUR
18 LIFE.

19 SO THIS MEF2 GENE IS RELATED TO A LOT OF
20 OTHER KIDS. A LOT OF KIDS ARE ASSOCIATED WITH THIS
21 NOW. AND AUTISM IS BECOMING BIGGER AND BIGGER EVERY
22 DAY. SO FOR THESE KIDS THERE'S A LOT OF RESEARCH, A
23 LOT OF THINGS ARE GOING ON, BUT IT'S JUST TALK. I'M
24 SORRY. I'M NOT TRYING TO BE DISRESPECTFUL, BUT I
25 DIDN'T SEE ANYTHING THAT'S BEEN DONE WITH THIS KID.

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1 IF WE GO ONE TIME, WHEN I TAKE MY SON THREE YEARS
2 OLD TO SCHOOL, I LOOK AT THE KIDS. SOMEBODY COMING
3 IN WHEELCHAIR, SOMEBODY CANNOT TALK, SOMEBODY CANNOT
4 SPEAK. I SEE THESE KIDS EVERY DAY. BEFORE I WAS
5 JUST LOOKING AT THEM. NOW IT COMES ON ME AND I'M
6 LOOKING AT MY KID GOING THROUGH THIS EVERY DAY.
7 IT'S A VERY HARD THING.

8 AND THESE KIDS ARE NORMAL KIDS LIKE ANY
9 OTHER KID. IF YOU LOOK AT THEM, THEY WANT TO COME
10 HUG YOU. THEY WANT TO LOOK AT YOU. THEY WANT --
11 THEY HAVE THE FEELINGS LIKE OTHER KIDS. WE JUST
12 NEED A CHANCE, AND THIS IS THE ONLY CHANCE THAT WE
13 HAVE WHERE WE HAVE DR. LIPTON. AND HE'S THE ONLY
14 PERSON IN THIS WORLD WHO'S WORKING ON THIS VERY BIG
15 RESEARCH HE'S DOING.

16 MY SON WENT TO BIOPSY LAST TWO MONTHS BACK
17 AT UC SAN DIEGO, AND THEY HAVE DONE -- WHAT DO YOU
18 CALL THIS? -- BIOPSY. THEY HAVE DONE ON HIM SKIN
19 BIOPSY FROM THERE. THEY SEND IT TO UC SAN DIEGO AND
20 HE IS GROWING THE CELLS. THERE'S SO MANY OTHER
21 KIDS ARE AFFECTED WITH THIS, YOU KNOW.

22 AS A PARENT, WE HAVE NOWHERE TO LOOK
23 BECAUSE I'M -- I WORK FOR COMMISSION. I MAKE HARDLY
24 ENOUGH MONEY TO SUPPORT MY FAMILY. MY WIFE AT HOME,
25 SHE'S TAKING CARE OF THE TWO KIDS. SO THIS IS THE

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1 ONLY PLACE WE CAN COME AND LOOK TO YOU GUYS AND TO
2 SEE IF SOMETHING CAN BE DONE FOR OUR KIDS LIKE US.
3 BY THIS GENE, DR. LIPTON, HE HAS DONE A LOT OF
4 THINGS IN THE PAST. IF HE CAN SUCCEED ON THIS, WE
5 CAN MOVE FORWARD AND SAVE A LOT OF KIDS AND THEY CAN
6 HAVE A NORMAL LIFE. THAT'S ALL. THANK YOU SO MUCH.

7 OPERATOR: LADIES AND GENTLEMEN, IF YOU
8 WISH TO ASK A QUESTION, PLEASE DO PRESS STAR THEN
9 ONE. WE DO HAVE DR. STUART LIPTON WITH US. PLEASE
10 GO AHEAD, SIR.

11 DR. LIPTON: STUART LIPTON, PROFESSOR AT
12 SCRIPPS RESEARCH INSTITUTE AT UCSD. I SUBMITTED A
13 DISC2 APPLICATION 11070 ON AUTISM AS YOU JUST HEARD.
14 WE RECEIVED A FUNDABLE SCORE OF 87. OUR TITLE IS
15 "DRUG DEVELOPMENT FOR AUTISM SPECTRUM DISORDER USING
16 HUMAN PATIENT IPSC'S."

17 I WANT TO THANK YOU ALL FOR THE HARD WORK
18 YOU'RE DOING IN REVIEWING THESE GRANTS. I
19 UNDERSTAND IT'S VERY DIFFICULT TO DECIDE WHO TO FUND
20 AND WHO NOT TO, BUT I HAVE TWO BRIEF COMMENTS IN
21 THIS REGARD.

22 FIRST, THIS IS THE ONLY APPLICATION IN
23 FRONT OF YOU, TO MY KNOWLEDGE, ON AUTISM. AND
24 AUTISM IS THE MOST COMMON DISEASE YOU ARE GOING TO
25 CONSIDER TODAY. WITH AN INCIDENCE OF ONE IN 59

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1 PERSONS, ACCORDING TO THE LATEST CDC FIGURES, AND
2 ACTUALLY HIGHER IN CALIFORNIA FOR REASONS THAT ARE
3 NOT CLEAR, THERE'S NO CURE OR TREATMENT.

4 SECOND, BY TOTAL SERENDIPITY, OUR GROUP
5 HAPPENED TO DISCOVER A GENE, AS YOU HEARD FROM THE
6 KAHN FAMILY, CALLED MEF2C. IT'S A PRINCIPAL DRIVER
7 OF AUTISM PHENOTYPES.

8 WE'RE STUDYING IPS CELLS FROM MANY
9 PATIENTS, MORE THAN A DOZEN PATIENTS, WITH MEF2C
10 HAPLO INSUFFICIENCY AND WITH OUR CRISPR-CAS9
11 CORRECTIONS AS CONTROLS. WE'RE USING THIS AS A
12 MODEL FOR AUTISM.

13 MOST IMPORTANTLY, MEF2C HAS BEEN SHOWN TO
14 BE THE TRANSCRIPTION FACTOR THAT DRIVES EXPRESSION
15 OF MANY OTHER AUTISM GENES. SO IT'S A TREATMENT FOR
16 THE MEF2C TYPE OF AUTISM, BUT WILL LIKELY TREAT MANY
17 OTHER FORMS OF AUTISM. IN FACT, WE'VE PUBLISHED
18 PRELIMINARY DATA IN *NATURE* SHOWING THIS TO BE TRUE.

19 NOW, THIS RELATIONSHIP OF MEF2C TO OTHER
20 FORMS OF AUTISM WAS ACTUALLY DISCOVERED AND
21 PUBLISHED IN TWO PAPERS PUBLISHED IN *CELL* AND
22 *SCIENCE* BY DANIEL GESCHLAND, M.D., PH.D., A
23 NEUROGENETICIST WHO YOU KNOW, THE DIRECTOR OF AUTISM
24 RESEARCH AT UCLA, AND WHO IS NOT IN ANY WAY
25 CONNECTED WITH THIS APPLICATION.

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1 DESPITE THIS FACT, I APPROACHED
2 DR. GESCHLAND, AND HE, EVEN THOUGH I'M AT A
3 DIFFERENT INSTITUTION, VOLUNTEERED TO WRITE YOU A
4 LETTER THAT YOU HAVE BEFORE YOU NOW DOCUMENTING THE
5 IMPORTANCE OF STUDYING SPECIFICALLY MEF2C AS A
6 THERAPY BECAUSE IT DRIVES SO MANY OTHER FORMS OF
7 AUTISM. HE URGES YOU TO FUND THIS APPLICATION.

8 FINALLY, SINCE SUBMITTING THE GRANT, WE'VE
9 RECEIVED A SMALL DONATION FROM FAMILIES WITH AUTISM.
10 AND WE USED THESE IPS CELLS TO SCREEN, AND FOUND
11 THAT I STOCKED HIS OLD DERIVATIVE CAPABLE OF PASSING
12 THE BLOOD BRAIN BARRIER THAT STIMULATES MEF2C
13 ACTIVITY. AS YOU PROBABLY KNOW, I STOCKED HIS OLD
14 DRUGS THAT HAVE PREVIOUSLY BEEN APPROVED BY THE FDA.
15 SO WE'RE STARTING BY REPURPOSING THOSE DRUGS AND
16 ALSO IMPROVING UPON THEM WITH OUR SCRIPPS CHEMISTS.

17 AS AN ACADEMIC THAT HAS ALREADY GAINED FDA
18 APPROVAL OF OTHER DRUGS, AS YOU MAY KNOW, MERZ
19 GROUP, WE DEVELOPED THE MULTIMILLION BILLION DRUG
20 NAMENDA FOR ALZHEIMER'S DISEASE, WE KNOW THIS IS A
21 FEASIBLE APPROACH AND LIKELY TO GAIN FDA APPROVAL.
22 WE HAVE NO OTHER FUNDS FOR THIS WORK ON AUTISM. IF
23 YOU DON'T FUND IT, THE PROJECT DIES.

24 SEVERAL FAMILIES ACTUALLY CONTACTED ME
25 ABOUT TALKING TO YOU TODAY. AFTER TALKING WITH THE

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1 KAHN FAMILY, I THOUGHT THEY BEST REPRESENTED THOSE
2 MANY OTHER FAMILIES, AND THEY'RE PRESENT IN OAKLAND.
3 THEY DROVE TO OAKLAND TO MAKE THIS PRESENTATION. I
4 THANK YOU FOR YOUR ATTENTION AND HOPE THAT YOU CAN
5 HELP THE MANY, MANY THOUSANDS OF FAMILIES WITH
6 AUTISM IN THIS STATE BY FUNDING THIS WORK. THIS IS
7 THE MOST COMMON DISEASE WE'RE TALKING ABOUT TODAY.

8 MR. SHEEHY: THANK YOU, DR. LIPTON.

9 OPERATOR: THE NEXT PERSON ON THE LINE
10 WILL BE TRACY GRIKSCHIEIT. PLEASE GO AHEAD.

11 DR. GRIKSCHIEIT: HI. THIS IS TRACY
12 GRIKSCHIEIT. I'M A PEDIATRIC SURGEON AT CHILDREN'S
13 HOSPITAL LOS ANGELES, AND I'M ALSO A SCIENTIST AT
14 CHILDREN'S HOSPITAL LOS ANGELES AND USC.

15 AS WE LOOK DOWN THE LIST OF GRANTS, I'M
16 THE FIRST GREEN ONE THAT WAS NOT FUNDED AT POSITION
17 4. IT'S DISC2-10979.

18 LIVER DISEASE AFFECTS 30 MILLION PEOPLE
19 WORLDWIDE. AND AS A PEDIATRIC SURGEON, I TAKE CARE
20 OF A LOT OF CHILDREN WHO HAVE A METABOLIC LIVER
21 DISORDER. LIVER DISORDERS IN CHILDREN ARE A VERY
22 RATIONAL PLACE TO START WITH CELL THERAPIES BECAUSE
23 WE ONLY NEED TO REPLACE 5 TO 10 PERCENT OF THE LIVER
24 IN ORDER TO HAVE THEM LIVE A FUNCTIONAL LIFE. AND
25 RIGHT NOW THESE CHILDREN WAIT FOR A LIVER TRANSPLANT

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1 AND THEN NEED TOTAL IMMUNOSUPPRESSION, AND THE GRAFT
2 USUALLY LASTS UP TO FIVE YEARS IF IT LASTS THAT
3 LONG, AFTER HAVING MULTIMILLION DOLLAR SURGERIES
4 THAT CAN RESULT IN HOSPITALIZATIONS FOR UP TO A 180
5 WEEKS. SO THEY CAN HAVE A VERY LONG
6 HOSPITALIZATION, AND IT'S ALSO VERY COSTLY WITH A
7 VERY POOR QUALITY OF LIFE.

8 THE UPLIFT PROPOSAL IS SPECIAL IN THAT WE
9 HAVE FORMED A COLLABORATION TO USE CELLS THAT ARE
10 UNIVERSAL, MEANING THAT THEY CAN EVADE THE BODY'S
11 IMMUNE SYSTEM. SO NOT ONLY DO WE WANT TO FIX LIVER
12 DISORDERS IN CHILDREN, BUT ALSO GIVE THEM A THERAPY
13 THAT WOULD NOT REQUIRE IMMUNOSUPPRESSION.

14 I UNDERSTAND THAT THERE MAY NOT BE MONEY
15 FOR THESE GRANTS RIGHT NOW, AND I'M GRATEFUL FOR THE
16 SUGGESTION THAT MAYBE THIS COULD STAY OPEN BECAUSE
17 WE REALLY WOULD LIKE TO HELP PEDIATRIC PATIENTS IN
18 LIVER FAILURE AND NOT COMMIT THEM TO A LIFETIME OF
19 IMMUNOSUPPRESSION OR A THERAPY THAT JUST DOESN'T
20 STAND UP OVER TIME.

21 IF THIS IS SUCCESSFUL, THERE ARE 30
22 MILLION MORE PEOPLE AROUND THE WORLD WHO COULD
23 BENEFIT.

24 MR. SHEEHY: THANK YOU, DR. GRIKSCHIT.
25 IS THERE ANY MORE PUBLIC COMMENT?

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1 OPERATOR: ONCE AGAIN, AS A REMINDER, IF
2 YOU WISH TO ASK A QUESTION, PLEASE PRESS STAR THEN
3 ONE.

4 SPEAKERS, CURRENTLY WE HAVE NO ADDITIONAL
5 QUESTIONS IN QUEUE. PLEASE DO CONTINUE.

6 MR. SHEEHY: I JUST WANT TO BE -- SO WHAT
7 IS THE IMPACT OF THIS RECOMMENDATION? SO DOES THAT
8 MEAN THAT IF WE VOTE FOR THIS RECOMMENDATION IN --
9 THIS MOTION IN ITS FORM, THAT THE ONLY CIRM FUNDS
10 THAT WOULD BE AVAILABLE WOULD BE BRIDGE FUNDS?

11 MR. TOCHER: GOOD QUESTION, JEFF. IT
12 DOESN'T PRECLUDE THE FUTURE ACT OF A BOARD TO
13 ALLOCATE SUCH FUNDS. IT JUST ENSURES THAT THE
14 OPPORTUNITY TO USE BRIDGE FUNDS THAT ARE
15 SPECIFICALLY TARGETED FOR THESE APPLICATIONS CAN BE
16 USED AND THAT THAT MAKES IT SORT OF EASIER FOR
17 FUTURE BOARD DISCUSSIONS TO OCCUR WITHOUT RECUSALS.

18 MR. SHEEHY: SO UNLESS WE HAVE ANY OTHER
19 DISCUSSION AT THE BOARD LEVEL, I THINK WE'RE READY
20 TO CALL THE ROLL.

21 MS. BONNEVILLE: ANNE-MARIE DULIEGE.
22 DAVID HIGGINS.

23 DR. HIGGINS: YES.

24 MS. BONNEVILLE: STEVE JUELSGAARD.

25 MR. JUELSGAARD: YES.

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1 MS. BONNEVILLE: DAVE MARTIN. LAUREN
2 MILLER.
3 MS. MILLER: YES.
4 MS. BONNEVILLE: DAVE MARTIN, IS THAT YOU?
5 DR. MARTIN: YES. YES, IT IS.
6 MS. BONNEVILLE: ADRIANA PADILLA.
7 DR. PADILLA: YES.
8 MS. BONNEVILLE: JOE PANETTA. FRANCISCO
9 PRIETO.
10 DR. PRIETO: AYE.
11 MS. BONNEVILLE: AL ROWLETT.
12 MR. ROWLETT: YES.
13 MS. BONNEVILLE: JEFF SHEEHY.
14 MR. SHEEHY: YES.
15 MS. BONNEVILLE: OS STEWARD.
16 DR. STEWARD: YES.
17 MS. BONNEVILLE: JONATHAN THOMAS.
18 CHAIRMAN THOMAS: YES.
19 MS. BONNEVILLE: ART TORRES.
20 MR. TORRES: AYE.
21 MS. BONNEVILLE: MOTION CARRIES.
22 MR. SHEEHY: GREAT. THANK YOU. I BELIEVE
23 THAT CONCLUDES THE BUSINESS OF THE APPLICATION
24 REVIEW SUBCOMMITTEE.
25 CHAIRMAN THOMAS: THANK YOU VERY MUCH,

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1 MR. SHEEHY. THAT CONCLUDES THE BUSINESS FOR TODAY
2 UNLESS THERE IS PUBLIC COMMENT OF A GENERAL NATURE
3 ON ANYTHING ELSE ANYONE WOULD LIKE TO SPEAK ABOUT.
4 DO WE HAVE ANY PUBLIC COMMENT EITHER HERE OR AT THE
5 OTHER SITES?

6 MR. REED: DO I UNDERSTAND CORRECTLY THAT
7 THIS VOTE DOES NOT PRECLUDE FURTHER FUNDING, IF
8 LOCATED, TO BE USED FOR THESE GRANTS?

9 MS. BONNEVILLE: CORRECT.

10 MR. REED: THE MEETING ONE WEEK FROM
11 TODAY, IS THAT IMPORTANT FOR THEM TO DRIVE UP AND
12 ATTEND TO MAKE THE PRESENTATION AGAIN, OR WHAT IS
13 YOUR OPINION ON THAT?

14 CHAIRMAN THOMAS: NO. WE'VE ALREADY GOT
15 THE AGENDA THAT'S PROPOSED FOR NEXT WEEK, DON. SO
16 WE'VE HEARD HERE, AND THIS IS A FULL MEETING OF THE
17 ICOC AS WELL AS THE APPLICATION REVIEW SUBCOMMITTEE.
18 SO THEY HAVE HEARD THEIR TESTIMONY. SO THEY DON'T
19 NEED TO COME NEXT WEEK FOR THAT.

20 MR. REED: THANK YOU.

21 MR. SHEEHY: BUT WOULD IT NOT BE TRUE THAT
22 AT THAT MEETING WITH THE BOARD'S DECISION THERE'S
23 THE POTENTIAL FOR -- IT'S NOT IMPOSSIBLE FOR OTHER
24 CIRM FUNDS TO BE ALLOCATED?

25 MR. TOCHER: THAT'S CORRECT.

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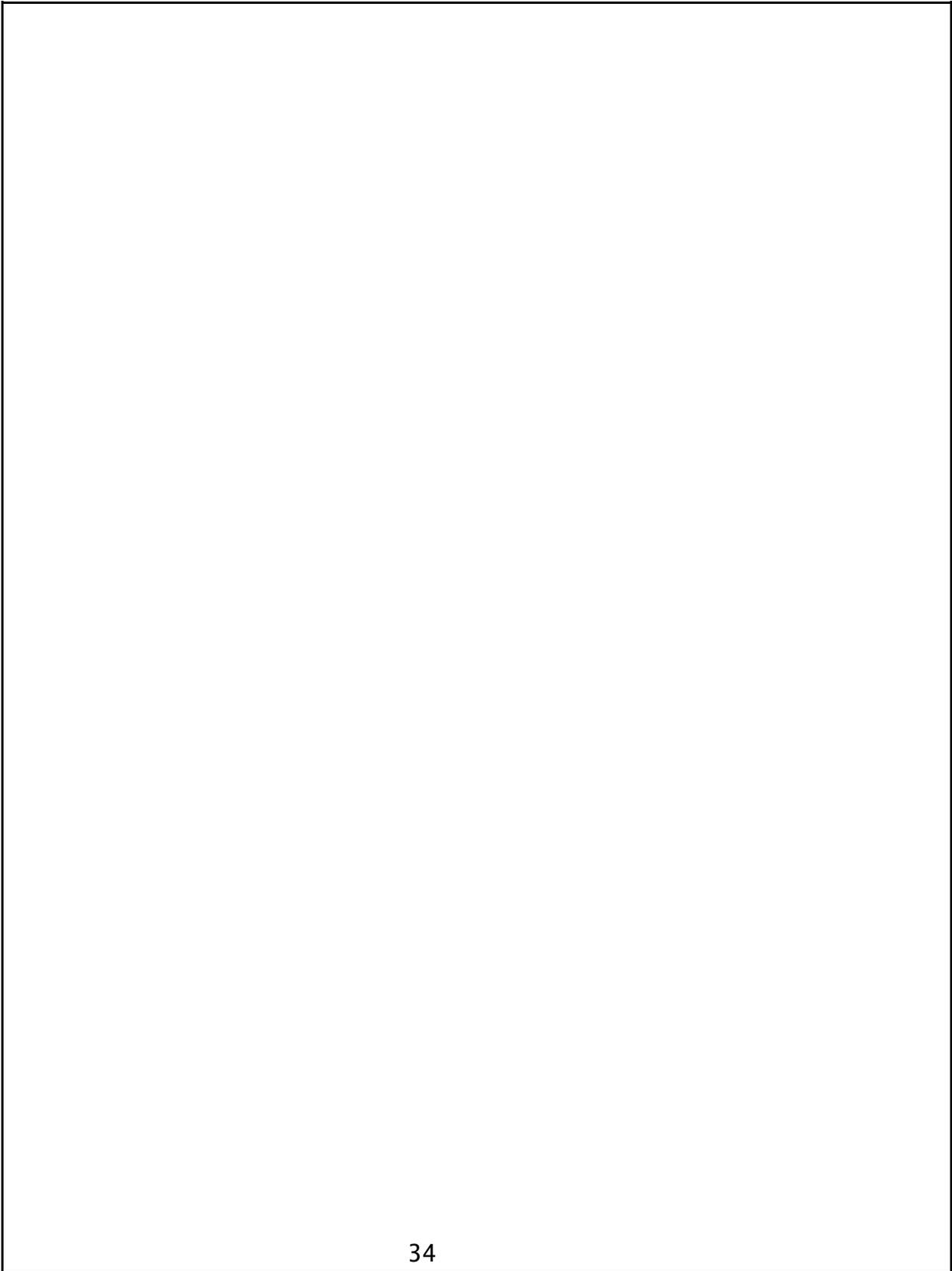
MR. REED: THANK YOU VERY MUCH FOR THAT
CLARIFICATION.

CHAIRMAN THOMAS: OKAY. I THINK THAT
CONCLUDES. HEARING NO FURTHER PUBLIC COMMENT, THAT
CONCLUDES TODAY'S MEETING. THANK YOU VERY MUCH,
EVERYBODY. WE STAND ADJOURNED.

(THE MEETING WAS THEN ADJOURNED AT 12:51 P.M.)

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REPORTER'S CERTIFICATE

I, BETH C. DRAIN, A CERTIFIED SHORTHAND REPORTER IN AND FOR THE STATE OF CALIFORNIA, HEREBY CERTIFY THAT THE FOREGOING TRANSCRIPT OF THE TELEPHONIC PROCEEDINGS BEFORE THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE AND THE APPLICATION REVIEW SUBCOMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON OCTOBER 11, 2018, WAS HELD AS HEREIN APPEARS AND THAT THIS IS THE ORIGINAL TRANSCRIPT THEREOF AND THAT THE STATEMENTS THAT APPEAR IN THIS TRANSCRIPT WERE REPORTED STENOGRAPHICALLY BY ME AND TRANSCRIBED BY ME. I ALSO CERTIFY THAT THIS TRANSCRIPT IS A TRUE AND ACCURATE RECORD OF THE PROCEEDING.

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